HEMATOLOGICAL DISORDERS—Cost Studies

PHM1

COST-EFFECTIVENESS OF ONCE-DAILY ORAL CHELATION THERAPY WITH DEFERASIROX VERSUS INFUSIONAL DEFEROXAMINE IN TRANSFUSION-DEPENDENT THALASSEMIA PATIENTS

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OBJECTIVES: Deferasirox is a recently approved once-daily oral chelator that has been shown to produce reductions in liver iron concentrations and serum ferritin similar to those with infusional deferoxamine. The cost-effectiveness of deferasirox vs deferoxamine in β-thalassemia major patients have has not been examined. **METHODS:** A Markov model was used to estimate the total additional lifetime costs and quality-adjusted life years (QALYs) gained with deferasirox versus deferoxamine in patients with β-thalassemia major and chronic iron overload from blood transfusions. Patients were assumed to receive prescribed dosages of deferasirox and deferoxamine that have been shown to be similarly effective in such patients. Compliance with deferoxamine as well as costs of deferoxamine administration and complications of iron overload were based on analyses of health insurance claims data of transfusion-dependent thalassemia patients. Probabilities of complications of iron overload and death by level compliance with chelation were estimated using data from published studies. Because data on compliance with deferasirox in typical clinical practice are unavailable, we used published data on compliance with the oral chelator deferiprone vs deferoxamine. Utilities (weights representing patient quality of life) were based on a study of patient preferences for oral vs infusional chelation therapy, as well as published literature and assumption. A US healthcare system perspective was employed. **RESULTS:** Deferasirox results in a gain of 3.9 QALYs per patient at an additional expected lifetime cost of $133,321 per patient. Cost-effectiveness is $33,792 per QALY gained. Cost-effectiveness is sensitive to the estimated costs of deferoxamine administration and the quality of life benefit associated with oral vs infusional therapy and is more favorable in younger patients. **CONCLUSIONS:** The cost-effectiveness of deferasirox vs deferoxamine in patients with transfusion-dependent β-thalassemia is within the range considered generally-accepted in the United States.

PHM2

ECONOMIC AND QUALITY OF LIFE BURDEN OF HIGH-RISK ACUTE LYMPHOBLASTIC LEUKEMIA

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OBJECTIVES: Patients with high-risk acute lymphoblastic leukemia (ALL), including Philadelphia chromosome positive (Ph+) ALL, typically have extremely poor prognosis, experience poor quality of life (QoL) and incur high economic cost. This study examined the economic and humanistic outcomes for high-risk ALL. **METHODS:** A systematic search of the English-language literature published between 1990 and 2005 was conducted. Additional searches were conducted from the retrieved article bibliographies and appropriate conference proceedings (2000–2005). Articles selected for inclusion were prospective or retrospective studies specifically designed to examine burden of illness, direct medical costs, cost drivers, or QoL outcomes of ALL and treatments. **RESULTS:** Of 798 abstracts screened, 106 met selection criteria and were reviewed in detail. Forty-nine and 47 studies focused on the economics and QoL aspects of ALL, respectively. The average annual direct medical cost per high-risk ALL patient ranged from $100,000 to $136,000. Hospitalization was the major cost component comprising 50%–80% of total direct costs. Major hospital cost drivers included infections, chemotherapy, growth factors, transfusions, and transplantation. These drivers resulted in more frequent hospitalizations and longer ICU lengths of stay for high risk patients. High-risk ALL patients typically had psychological problems and physical complaints, especially in domains of emotion, cognition, and pain. Furthermore, high-risk patients were more likely to have poorer QoL than standard-risk patients due to higher relapse rates and increased need for transplantation. **CONCLUSIONS:** ALL exacts a substantial economic and humanistic burden on patients, their loved ones and society in general. This burden appears particularly heavy for high-risk patients, such as Ph+ ALL. Imatinib, a molecularly targeted therapy, has been reported to prolong disease-free-survival in Ph+ ALL with good tolerability in clinical studies. Research is warranted to evaluate the economic and humanistic benefits of imatinib as compared to the current therapies in the treatment of Ph+ ALL.

PHM3

ECONOMIC ANALYSIS OF RECOMBINANT ACTIVATED FACTOR VII IN THE HOME TREATMENT OF MINOR-TO-MODERATE BLEEDS IN HEMOPHILIA PATIENTS WITH INHIBITORS: A U.S. COST-OF-BLEED MODEL

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OBJECTIVES: To compare the cost of treatment for three "on-demand" treatment regimens using recombinant activated Factor VII (rFVIIa [NovoSeven®]) and activated prothrombin-complex concentrate (APCC [FEIBA® VH]) for home treatment of minor-to-moderate bleeds in hemophilia with inhibitors. **METHODS:** A decision analytic model was developed from the payer's perspective to calculate the projected cost per bleeding episode and the 1-year cost of treatment for three "on-demand" treatment strategies consisting of first, second, and third-line treatments: rFVIIa/rFVIIa/rFVIIa, APCC/rFVIIa/rFVIIa, and APCC/APCC/rFVIIa. Published literature was used to define treatment algorithms, number of bleeds, dosing, costs, efficacy, and re-bleeds. Evaluable bleeds controlled with rFVIIa and APCC ranged from 88–93% and 78–81%, respectively. Number of bleeds was assumed to be 15 per year (range 10–20). Drug costs were based on 2005 U.S. average wholesale prices; other direct medical costs reflected 2005 values. Univariate and probabilistic sensitivity analyses (PSA) were conducted on key variables to ascertain model robustness. **RESULTS:** The cost per bleed (and 1-year cost of treatment) per patient was $28,076 ($421,137), $30,883 ($463,251), and $32,150 ($482,253) using rFVIIa/rFVIIa/rFVIIa, APCC/rFVIIa/rFVIIa, and APCC/APCC/rFVIIa, respectively. Annual cost offsets ranging from $42,115–$61,116 per patient occurred for the rFVIIa-only regimen through avoidance of second and third lines of treatment. Univariate sensitivity analyses showed consistent results with the base case. In PSA, the rFVIIa-only strategy was less expensive than either alternative in 68% of 10,000 model simulations. **CONCLUSIONS:** The management of minor-to-moderate bleeds extends beyond the initial line of treatment, and should include the economic impact of rebleeding over multiple lines of therapy. The annual cost of treatment for minor-